

New Technology for the Derivation of Human Pluripotent Stem Cell Lines for Clinical Use

Grant Award Details

New Technology for the Derivation of Human Pluripotent Stem Cell Lines for Clinical Use

Grant Type: New Cell Lines

Grant Number: RL1-00667

Investigator:

Name: Martin Pera

Institution: University of Southern California

Type: PI

Human Stem Cell Use: Embryonic Stem Cell, iPS Cell, Other

Cell Line Generation: Embryonic Stem Cell, iPS Cell, Other

Award Value: \$1,266,134

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 3

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Grant Application Details

Application Title: New Technology ForThe Derivation of Human Pluripotent Stem Cell Lines For Clinical Use

Public Abstract:

Since their discovery almost ten years ago, there has been steady progress towards the application of human embryonic stem (ES) cells in medicine. Now, the field is on the threshold of a new era. Recent results from several laboratories show that human skin cells can be converted to cells resembling ES cells through simple genetic manipulations in the laboratory. There is currently much excitement about these induced pluripotent stem (iPS) cells, which might have advantages over ES cells in studying and treating disease. However, we do not yet sufficiently understand their nature and potential to be certain that they can replace (ES) cells in research and therapy.

Because of this, it is important to continue to develop new ES cell lines, and to compare their properties with those of iPS cells. Technological advances in ES cell research now enable us to grow stem cells under conditions that are much more suitable for future patient use than those used to develop the first ES cell lines. However, these new methodologies have for the most part been developed with, and tested on, a handful of the long-established ES cell lines on the NIH Registry.

In this proposal, we will rigorously test improved techniques for growing stem cells, developed in our lab and elsewhere, to see how well they work in deriving new ES cell lines. We will ensure that these new methods allow for production and maintenance of ES cells free of genetic abnormalities. Current technology for producing iPS cell lines also has significant limitations. Published procedures run the risk of producing genetic damage in the stem cells, and rely on the use of cancer causing genes. Also, using current techniques it is more difficult to produce iPS cells from adult tissue (the most appropriate source for therapy) than from fetal tissue. This proposal examines alternate approaches to generating iPS cells aimed to circumvent these limitations.

Using these improved technologies, we will carefully compare the properties of ES cells and iPS cells derived and propagated under identical conditions. This is important, because to find out if iPS cells really are equivalent to ES cells, we have to compare examples of both cell types made and grown under the same conditions. Otherwise, differences that we see may be related to how the cells were produced and propagated, or how long they have been grown in the laboratory, rather than to inherent differences between them.

The significance of this research is threefold. First, new ES cell lines suitable for therapeutic use will be derived from embryos and made available to any researcher who requires them. Second, we will answer the important scientific question over the equivalency of ES and iPS cells under experimental conditions that remove variables related to cell culture techniques and cell age. Finally, we will improve and validate the technology for derivation and maintenance of ES and iPS cells for future clinical use.

Statement of Benefit to California:

Through their support of Proposition 71 the citizens of California recognized the fundamental importance of stem cell research to the future of biotechnology and regenerative medicine. Funding from this initiative enables California scientists to work outside of limits imposed on federally funded research, to develop and study new pluripotent stem cell lines from spare embryos. These embryonic stem (ES) cell lines have the property of pluripotency, or the ability to give rise to any type of body cell. Now, rapid technological progress in methodology for growing embryonic stem cells, and for developing cells with the properties of embryonic stem cells from adult tissue, provide new opportunities for producing stem cell lines that are safe for patient use, or for producing cell lines that will avoid the problems of tissue rejection during transplantation. However, in many ways these new technologies are as yet untested. This proposal is aimed at applying and assessing new methods for making pluripotent stem cells from embryos or from adult tissue. We will capitalize on our extensive experience in deriving and characterizing stem cell lines, to achieve three goals: first, the development of new cell lines under conditions that ensure they will be as safe as possible for use in patients; second, development of improved techniques for making pluripotent cell lines from embryos or adult tissues; third, a clear answer to the key question of whether stem cells from adult tissues are equivalent in potential to those from embryos. These advances will ensure that the best possible stem cell lines are available to Californian scientists, physicians and their future patients. Through this program, stem cell research in the State will remain at the forefront of the field and will lead in technological innovation in cell culture biotechnology, as well as in basic science and translational and clinical research. Finally the ability to derive new cell lines from embryos and carefully compare them to cell lines from adult tissue will provide data that is critical to the future of the field and to directing the research efforts of the California Institute for Regenerative Medicine.

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